Promise of gene therapy to treat sickle cell disease.

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Public Summary:
New methods to treat sickle cell disease by gene therapy and gene editing are described.

Scientific Abstract:
INTRODUCTION: Sickle cell anemia (SCA) is a hereditary blood disease caused by a single-gene mutation that affects millions of individuals world-wide. In this review, we focus on techniques to treat SCA by ex vivo genetic manipulation of hematopoietic stem/progenitor cells (HSPC), emphasizing replacement gene therapy and gene editing. AREAS COVERED: Viral transduction of an anti-sickling beta-like globin gene has been tested in pre-clinical and early-phase clinical studies, and shows promising preliminary results. Targeted editing of endogenous genes by site-directed nucleases has been developed more recently, and several approaches also are nearing clinical translation. EXPERT OPINION: The indications and timing of gene therapy for SCA in lieu of supportive care treatment and allogeneic hematopoietic cell transplantation are still undefined. In addition, ensuring access to the treatment where the disease is endemic will present important challenges that must be addressed. Nonetheless, gene therapy and gene editing techniques have transformative potential as a universal curative option in SCA.